Biotechnology: Delivering on the Promise

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The research and development of innovative new therapies are expensive and risky processes, and companies require substantial resources if they are to translate basic science into modern biomedical products. When resources are limited, as in the current economic climate, biotechnology startup companies are often among the first casualties in the fight for assets. Policy-makers can play an important role in bolstering these important innovators.

The biotechnology industry engages in the research and development of innovative health care (as well as agricultural, industrial, and environmental) technologies. Biotechnology companies range from entrepreneurial startups that are developing a first product to Fortune 100 multinational companies. These research-intensive companies strive to develop modern biomedical products for patients around the world, but the science of biotechnology isn’t easy. Nature does not readily yield her secrets. Still, every day, in nearly every country, researchers decode a bit more of the language of life, and the science continues to astonish and amaze. But if biotechnology companies are to translate this science into modern medicines, diagnostic tools, and techniques, resources are critical. During these hard economic times, policy-makers can play an important role in bolstering the biomedical industry.

Today, there are more than 250 biotechnology products and vaccines approved by the U.S. Food and Drug Administration (FDA) that extend lives and provide new hope for patients living with debilitating diseases (1). And more than 600 new biotechnology medicines are currently either in clinical trials or under review by the FDA for a variety of diseases, including cancer, Parkinson’s disease, diabetes, and more than 100 other conditions (2). Many other potential treatments, vaccines, and diagnostics are in earlier phases of development and, together with cutting-edge research in areas such as regenerative medicine, gene therapy, epigenomics, and synthetic biology, are expected to yield more effective ways to treat, preempt, and prevent disease than are possible today.

BIOTECHNOLOGY BLUES

To continue the move from the promise of basic research to the reality of breakthrough medicines and diagnostic tools, biotechnology companies need creative scientists, patient investors, and a policy environment that encourages innovation. Researching and developing new therapies is a long, expensive, and risky process. On average, the development of a new drug costs ~$1.2 billion over the course of 10 to 15 years from the time research begins until the drug receives FDA approval and is prescribed for patients (3). This price tag includes the cost of research dead ends and failed drug candidates along the way. For example, only one in three biologics—therapeutic drugs or vaccines that are produced by living organisms—makes it through the clinical trial process (3).

The ongoing financial downturn has left many biotechnology companies unable to access the investment capital they need to continue work on promising science. This situation has forced dozens of companies to shelve or delay projects, lay off workers, and, in some cases, close their doors (4). As of September 2009, at least 40 U.S. public biotechnology companies had either placed drug development programs on hold or cut them all together. These programs include therapies for HIV infection, cervical cancer, multiple sclerosis, and diabetes (4).

Approximately 85% of biotechnology companies are not yet profitable (5). These companies depend on investor capital and other financing sources to fund their R&D efforts. The current economic environment has hit such early-stage companies particularly hard. As of 1 December 2009, 39% of publicly traded U.S. biotechnology companies had only enough cash on hand to fund 1 year of operations, and 27% had only 6 months’ worth of cash in reserves (4).

Many companies in need of immediate funds are finding the capital markets closed to them. In a recent joint study by the Biotechnology Industry Organization (BIO) and Thompson Reuters (6), more than 80% of biotechnology investors surveyed acknowledged that the economic crisis has caused them to change their investment approach, placing an increased focus on a company’s cash position. For many investors, funding early-stage scientific research is now seen as too high-risk relative to other investment opportunities.

If biotechnology companies are forced to halt or delay promising projects for lack of funding, or worse, close their doors and abandon ideas forged from their accumulated research, we risk losing the benefits of medical advances that can save and improve lives and help create high-wage innovation economy jobs. Furthermore, we waste the time and resources spent to perform intricate research that subsequently must be discontinued.

Despite the current economic challenges, the long-term prospects for biotech remain strong. Companies continue to innovate, developing “miracle medicines” and new tools that allow more personalized and efficient care. Investors continue to believe in the basic business model as well. Fifty-seven percent of the investors surveyed in the Thomson Reuters–BIO study expected biotechnology to rebound by the end of 2009, and another 30% predict a rebound in 2010. To date, their confidence appears to have been borne out, with the NASDAQ Biotechnology Index rising more than 10% from the beginning of 2009 through 1 December 2009.

PROMISING RESEARCH MEETS SMART POLICY

Even gifted scientists engaged in cutting-edge research in well-run business settings require help from officials, whom they depend on to make policy that values and incentivizes innovation. Successful scientists with proven track records know how to innovate, but biotechnology innovation has always been a high-risk enterprise and always will be. Policy-makers can use their power to help improve the odds. We use the U.S. system of government to illustrate examples of policy changes that would spur the biotechnology sector.

Inventive incentives. First, the U.S. Congress can increase investor confidence by completing its work on health care re-
form and adopting an approach that lowers costs and increases access to quality health care while preserving incentives for innovation. The debate over the broad shape and fine details of health care reform has been lengthy and vigorous. Although careful deliberation is certainly appropriate for such an important initiative, uncertainty about the outcome is keeping cautious investors on the sidelines until the final shape of health care reform is clear. BIO and its members support the goal of universal health care coverage. We believe that every man, woman, and child in the United States—indeed, in the world—should have access to quality health care, including our most innovative products. This includes access to therapies that have not yet been developed and will not be developed without adequate incentives to invest in future biomedical R&D.

Spending on prescription medicines accounted for only 10.1% of the more than $2.2 trillion in national health spending in 2007 (7). Most of the long-term growth in health care spending is the result of increases in hospital care and physician and clinical services, whereas spending on prescription drugs has contributed only modestly to the growth in health care spending (8). Health reform legislation simply focused on ratcheting down reimbursement for medicines will not significantly reduce health care spending, but will make it more difficult for companies to attract the capital needed to develop the next generation of groundbreaking treatments.

Biopharmaceutical innovation offers some of the most compelling opportunities to improve treatments, save costs, and address unmet health needs (Fig. 1). Biotech innovation is essential to fighting chronic disease, which is both the leading cause of death and disability in the United States and the number-one driver of our country’s escalating health care costs. (9). Encouraging biomedical innovation must be at the heart of health care reform if it is to succeed.

Regulatory rehaul. Enacting a sound regulatory pathway for biosimilars—new versions of already approved therapeutics—is another important step that Congress can take to bring greater stability to the biotechnology sector. A balanced pathway to biosimilars will give biotech investors greater confidence in taking the financial risks of funding the research of biotech startups, knowing that if a new product is successfully developed, there will be a sufficient opportunity to recover their investment. At the same time, opening the market to competition from biosimilars, which are sometimes inaccurately referred to as biogenerics, can broaden access to and reduce the cost of cutting-edge biotechnology-derived drugs, helping to achieve one of the primary goals of health care reform. Given the complexity of biologics, a properly constructed biosimilars regulatory pathway must provide necessary protections to ensure patient safety and also must preserve the incentives for innovator companies that pursue the development of new breakthrough biomedical products.

As of December 2009, the U.S. House of Representatives has passed and the Senate innovation. Both versions of the U.S. health care reform bill employ a common-sense approach similar to the process and time-line currently in place for generic versions of chemical-based medicines such as aspirin and statins under the Hatch-Waxman Act. The average effective patent life on small-molecule or chemical-based drugs is 12.8 years once they are approved (10), whereas on average, generic competition begins for small-molecule drugs 13.5 years after FDA approval (11). The language under consideration by Congress would follow this model and maintain parity with small-molecule makers by allowing the original developer of a biologic to protect the proprietary data used to develop the medicine for at least 12 years. A shorter exclusivity period would prematurely deprive biotech innovators of their intellectual property and biotech investors of adequate time to gain a return on their investments (12). Establishing an effective and fair regulatory pathway for biosimilars that will lower costs and increase the number of patients who have access to pharmaceuticals will give investors confidence in backing biomedical innovation.

Financial lifeline. The enacting of health care reform that has innovation promotion as one of its focuses and the establishment of a balanced biosimilars approval pathway will improve the policy environment for innovation and the investment climate for the biotechnology sector, but many companies need more direct and immediate relief. Health care reform legislation being considered by the U.S. Senate would bring that relief by creating a therapeutic discovery project tax credit (13). Specifically, this proposal would reimburse small biotechnology companies with 250 employees or fewer for a portion of resources spent on therapeutic development activities, including hiring scientists and conducting clinical studies. The amendment authorizes $1 billion in credits or grants in the 2009 and 2010 tax years to pay for 50% of qualifying drug R&D activities. Eligible projects would be those designed to treat or prevent diseases or conditions by conducting preclinical
studies, clinical trials, or research protocols for the purpose of securing approval of a drug or biologic; identifying disease-related molecules for molecular diagnostics to guide therapeutic decisions; or developing processes, technologies, or products to enhance the delivery of therapeutics.

In granting these credits, the U.S. Treasury Department would support projects that are likely to lead to new therapies to treat areas of unmet medical need, address chronic or acute diseases, reduce long-term health care costs, or significantly advance President Obama’s challenge to cure cancer in our lifetime. Because biotechnology companies are at the forefront of all of these endeavors, the research tax credit can potentially benefit a broad range of companies. The research budgets of most companies that would qualify for such tax credits fall in the range of $10 million to $30 million per year, so the $1 billion allocated for the credits can cover many recipients. For the vast majority of small biotechnology companies that are not yet profitable, the credits can also be converted into grants (13).

These policy prescriptions can provide relief for some of the ills facing the industry and help sustain biotechnology researchers and their investors so that they can continue to collaborate on creative biomedical products. Millions of people are waiting, some for a new treatment, some for a cure. Biotechnology provides the best chance we have to end the waiting and deliver on the promise of modern biomedical innovation.

REFERENCES AND NOTES
4. BIO, unpublished data analysis.
5. BIO, unpublished analysis of data from FactSet Research Systems, Inc. database.
13. H.R. 3590, Patient Protection and Affordable Care Act (Amendment in Senate), Section 9023.